Pharmaceutical Management Branch
Cancer Therapy Evaluation Program, DCTDC, NCI

The Treatment Referral Center
and Non-Research (Compassionate) Use of Investigational Agents

Treatment Referral Center:

The Treatment Referral Center (TRC) is a means for the NCI to provide information to community oncologists and other health care professionals about therapeutic options for cancer patients. The TRC uses several resources (Physicians Data Query (PDQ), CTEP-Information System (CTEP-IS), to maintain a referral list of the most current active research protocols. First priority will be given to referring patients to Cooperative Group or Cancer Center trials. If a patient is unable to participate on a clinical trial then a non-research mechanism might be considered. Health care professionals may contact the Treatment Referral Center regarding therapeutic options, either clinical trials or non-research programs, by phone (301) 496-5725 or fax (301) 402-4870.

Non-Research (Compassionate) release:

The three mechanisms for providing an agent on for non-research use are, Special Exception, Group C and Treatment Referral Center Protocols. These mechanisms differ in purpose and in reporting and procedural responsibilities of the investigator. The ultimate purpose of the non-research (compassionate) program is to make non-approved agents, that have significant activity against specific malignancies, available to cancer patients and investigators. Investigators must consider the following questions when requesting an agent for non-research use:

- Is the patient ineligible for a research protocol?
- Have standard therapies been exhausted?
- Is there objective evidence that the investigational agent is active in the disease for which the request is being made? CTEP usually requires published phase II data as objective evidence.
- Is the drug likely to benefit the patient?

Request for non-research use may be considered if the answer to all these questions are affirmative.

Special Exception:

The Special Exception mechanism is the functional equivalent of a emergency IND but differs from it in that any registered investigator may obtain an agent directly from CTEP, instead of having to obtain an IND from the FDA. The following information is required to consider a Special Exception request; patient identifier (name or ID #), age, sex, diagnosis, previous cancer therapy, current clinical status, intended dose and schedule of the requested agent (this should be based on the currant literature), potential concomitant therapy, and pertinent laboratory data. Each request is reviewed and approved on a case by case basis.

Group C/Treatment IND:

Investigational agents that have been given Group C/Treatment IND designation by the FDA have reproducible efficacy in one or more specific tumor types. Such an agent is likely to alter the pattern of treatment of the disease and can be safely administered by properly trained physicians without specialized supportive care facilities. If a drug meets these criteria CTEP may initiate a formal application to the FDA to authorize Group C distribution for a specific indication. Such approval is not equivalent to formal FDA approval of effectiveness for this indication. Any registered investigator may receive a Group C agent.

Current Group C agents:

Agent	Indication
5-azacytidine	Refractory Acute Myelogenous Leukemia.

Treatment Referral Center (TRC) Protocols:

The NCI may make investigational treatments available via a TRC protocol for certain high priority agents or diseases. These protocols are offered to the NCI designated Clinical and Comprehensive Cancer Centers. All patients enrolled on a TRC protocol must receive their investigational therapy at an NCI designated Cancer Center.

Current TRC protocols: None

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